‘We’ve got miraculous therapies for ‘bubble boy disease’ that don’t fit in anyone’s business model’: Mainstream care elusive for most patients because of high costs

[Hataa?ii Tiisyatonii “HT” Begay] was born with a form of severe combined immunodeficiency, or SCID, which meant he had virtually no immune defenses. About 70 children are born with SCID each year in the United States and Canada, though only two or three will have the same type as HT. Without treatment, children with SCID typically die in the first two years of life.

Two and a half months later, at the University of California at San Francisco Benioff Children’s Hospital, HT became the first person in the world to receive an experimental gene therapy designed to build his immune system, cell by cell. The outcome of this experiment was far from certain — a big leap of trust for his family members, who were relying on Western medicine and traditional knowledge to find a path for HT to come home.

…

“We’ve got these transformative, miraculous therapies that don’t fit in anyone’s business model,” said Donald Kohn, a professor of microbiology, immunology and molecular genetics at the University of California at Los Angeles who has been treating patients with the experimental therapy, with dwindling funds. “It’s at a paradoxical state. It’s hard to get a research grant when you’ve kind of proven it’s effective.”

Researchers remain hopeful, and are spitballing new models. A nonprofit might be able to develop such medicines. They’re exploring the steps toward setting up commercial manufacturing, looking for alternative funding sources and talking to regulators.

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